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Backing visionary entrepreneurs

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Cell and Gene Therapy Symposium

Brussels, Oct 27, 2023

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EIC Programme Manager for Health and Biotech

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Objectives of the EIC Cell and Gene Therapy Symposium

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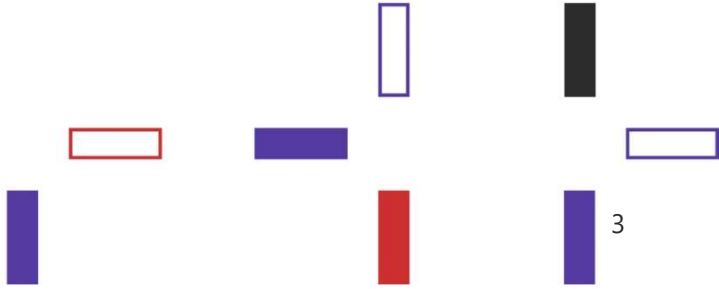
- **To inform** specialists in the field (ESGCT) and the public about the EIC Pathfinder Challenge-based Cell and Gene Therapy (CGT) portfolio
- **To demonstrate** the power of the EIC Pathfinder CGT portfolio: Why do we believe that the CGT portfolio can make the difference as vehicle/mechanism to achieving both, critical mass and strategic technological autonomy for Europe in the targeted areas (cell therapy manufacturing and gene delivery systems) in the short/medium and medium/long run, respectively
- **To promote** networking opportunities between the EIC CGT family and experts in the field and stakeholders (reception at the EIC premises)



EIC CGT portfolio

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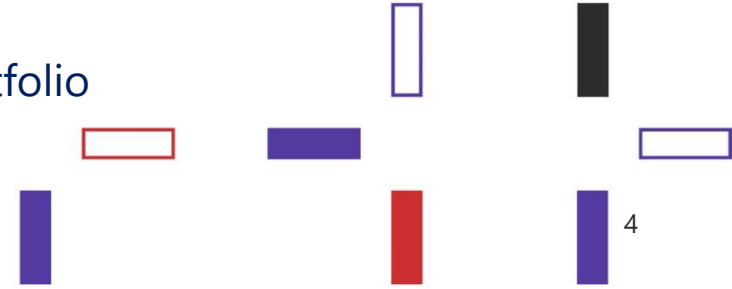
To accomplish that we'd need to share with you:

- What is the EIC Pathfinder CGT portfolio comprised of 2 well defined sub-portfolios
- Why we created the EIC CGT portfolio (a coherent set of selected EIC-funded projects)
- How we established the EIC CGT portfolio (shared component model)
- The 3 targeted areas of work at portfolio level: Technology, Regulatory and Exploitation
- How do we work at portfolio level for one year now (Panel discussion).
- Why we have invited the entire EIC CGT family in this Symposium
- Why EIC is strategically positioned well with its 2021 CGT portfolio



The EIC CGT portfolio is the outcome of the HORIZON-EIC-2021-PATHFINDERCHALLENGES-01-03

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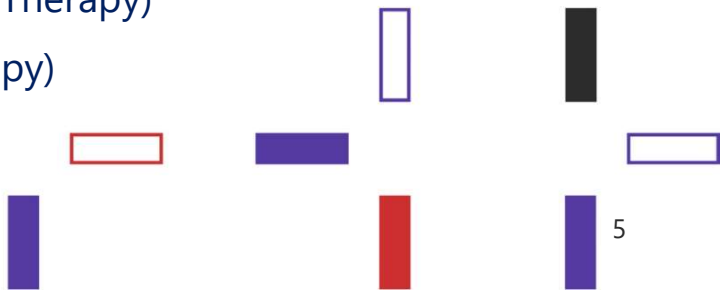
1. **AiPSC** (AI-powered platform for autologous iPSC manufacturing)
2. **SMARTER** (Smart manufacturing for autologous cell therapies enabled by innovative biomonitoring technologies and advanced process control)
3. **MUTAVAC** (Targeting cancer with mutanome based stem cell vaccine)
4. **T-FITNESS** (FINE-TUNING T CELL NETWORKS OF EXHAUSTION BY SYNTHETIC SENSORS)
5. **X-PAND** (Exploiting ex vivo expansion and deep multiomics profiling to bring novel, efficient and safer hematopoietic stem cell gene therapies to clinical application)
6. **PAT4CGT** (Automated online monitoring & control to improve processes and decision making in cell and gene therapy manufacturing)
7. **EdiGenT** (New Prime Editing and non-viral delivery strategies for Gene Therapy)
8. **AAVolution** (Next-generation AAV vectors for liver-directed gene therapy)
9. **NOVISTEM** (Non-VIral gene modified STEM cell therapy)



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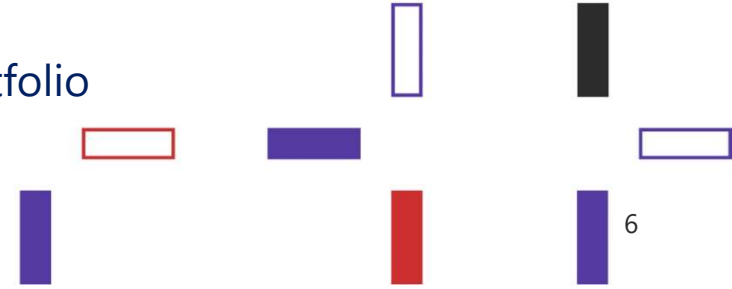

Market outlook 2022: The FDA approved only 11 gene therapies in the US market with over 1400 clinical trials running in 2021

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Challenges

- Safety
- In vivo efficacy
- Manufacturing/Scaling up

Multiple and complex challenges in Gene Therapy manufacturing

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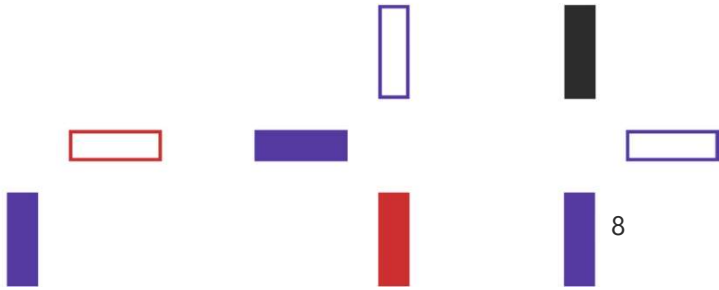
Despite the huge CGT potential, the market remains small with only a handful of approved products because of challenges such as:

- Lack of maturity in the manufacturing techniques:
  - For instance, in the AAV-based gene therapy: the ability to manufacture and deliver cellular production of high titers of viral vectors, high quality/purfication, and efficacious product (not all AAV particles contain the intended genetic payload.)
- Process standardization,
- Scaleup, cost-effective platform solutions and
- Lack of innovation in advanced manufacturing



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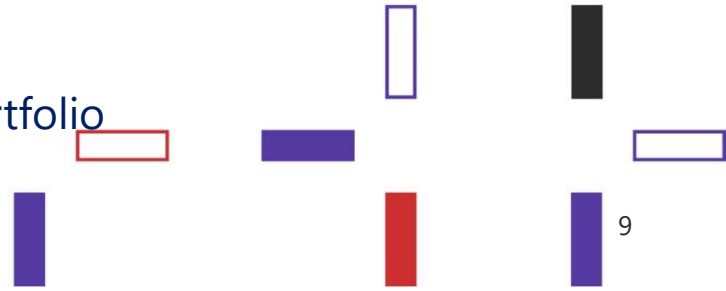

Creation of a coherent portfolio of CGT projects

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- For the creation of a coherent portfolio of CGT projects to be funded, the EIC PM and PA proposed to the evaluation panel the shared component logic/model. Detailed discussion among the 25 Panel members followed and finally, the Panel endorsed the approach and the selection of proposals. According to this model, the evaluation panel applied the following portfolio considerations:
    - All proposals were mapped against well-defined categories/ building blocks
    - The "**Shared component**" was identified based on one or more building blocks that are common to several proposals coming from different building blocks

- Soon after the selection of the nine CGT projects to establish the CGT portfolio and be funded under the EIC Pathfinder CGT Challenge, it became evident that two distinct sub portfolios would better serve the "shared component" logic/model. As a result, two sub-portfolios emerged:
    1. Advancing cell therapy manufacturing and products to a clinical stage
    2. Improving the effectiveness and lowering the risks of gene delivery systems (vectors)

EIC CGT portfolio Over

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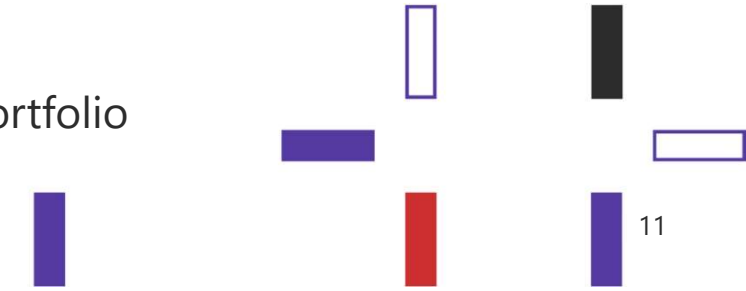

Target Areas of action at Portfolio level

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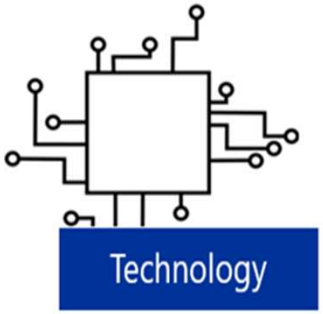
Portfolio vs project activities: Unlike the individual project, portfolio activities are taking place between 2 individual or a subgroup of projects within the CGT portfolio. Main goals to be achieved with the portfolio activities include:

- Tackle challenges of common interest in any of the three areas shown above
- Leverage opportunities of common interest in any of the above three areas
- Amplify the impact of the portfolio and on the portfolio so that the impact of an activity is more than the sum of the activities performed independently by each individual project



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EIC Pathfinder portfolio: Emerging technologies in CGT

Sub-portfolio 1: Advancing cell therapy manufacturing and products to a clinical stage

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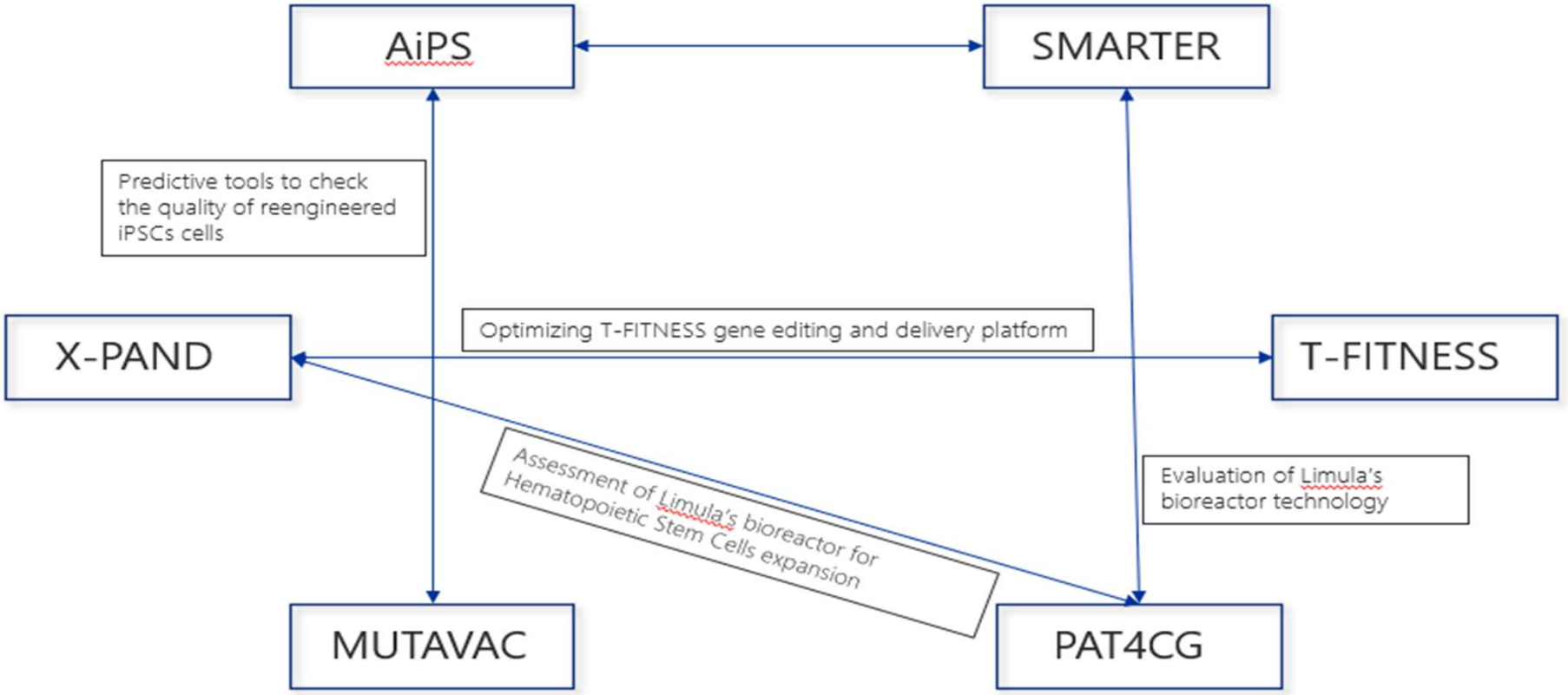

EIC Pathfinder portfolio: Emerging technologies in CGT
Sub-portfolio 2: **Improving the effectiveness and lowering the risks of gene delivery systems (vectors)**

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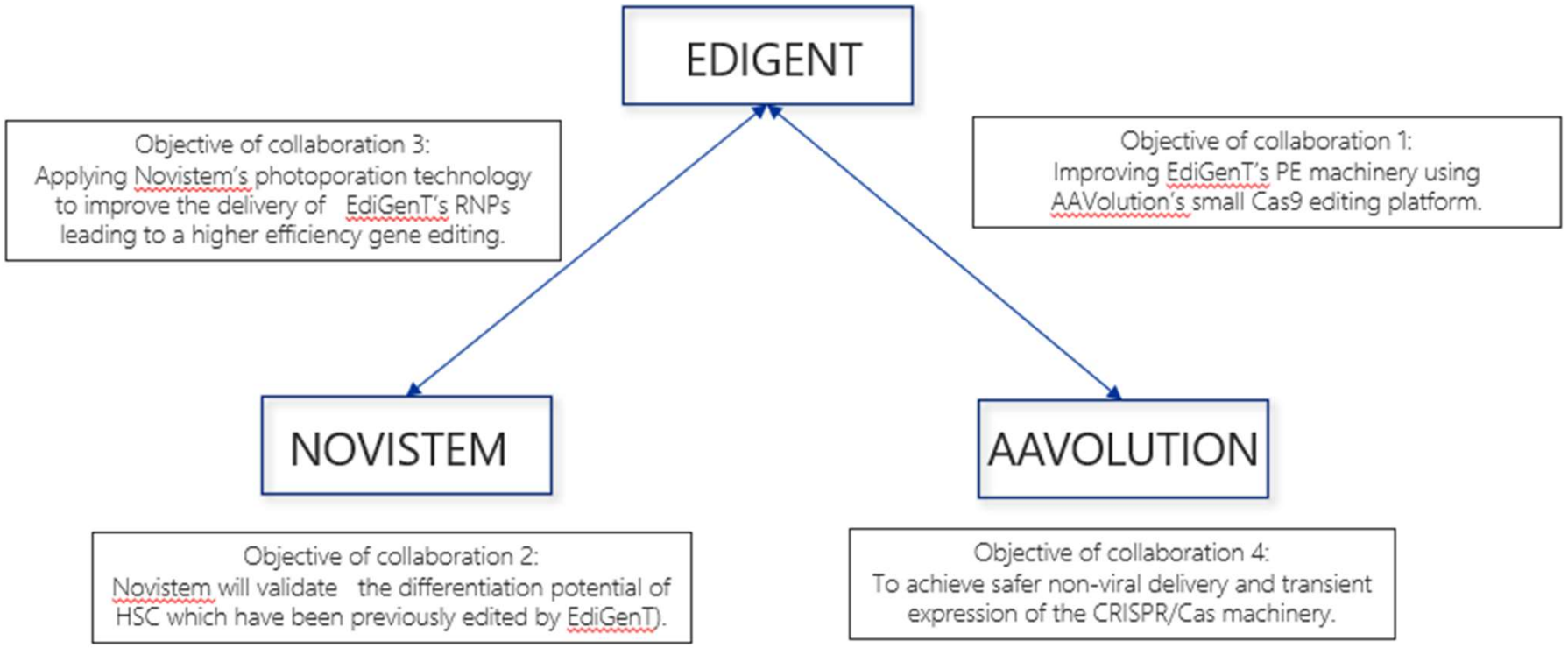

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EIC Accelerator biotech SparingVision strategic collaboration with Intellia Therapeutics

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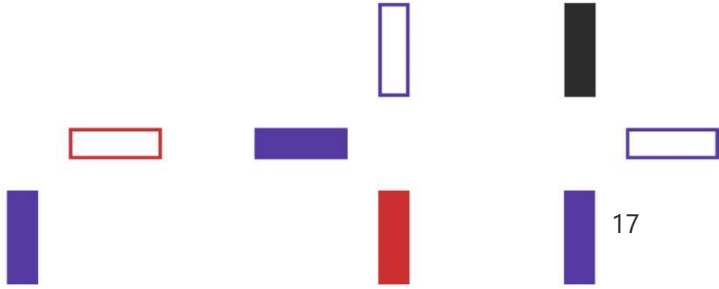
The case of the EIC Accelerator SME **SparingVision** a clinical-stage gene therapy company developing vision-saving treatments for ocular diseases, announced that it has selected a second target as part of its strategic collaboration with **Intellia Therapeutics**, Inc. (NASDAQ: NTLA) to develop novel genomic medicines utilizing CRISPR-based gene editing technologies for the treatment of ocular diseases (Paris Sept. 26, 2023).



$100M+ investments in North America (Jan. 1-May 25, 2023) align with the focus of the EIC CGT portfolio

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Company	Amount raised	Date	Technology	Lead Investors	EIC Health & Biotech portfolio corresponding to the investment
ElevateBio	$401M	5/24	Cell & Gene Therapy accelerator	Matrix	EIC Path Cell & Gene Therapy (CGT) portfolio
ReNAgade	$300M	5/23	RNA medicines	MPM, F2 Ventures	EIC Transition RNA-therapies portfolio
Cargo	$200M	3/1	CAR-T therapies for cancer	Third Rock, RTW, Perceptive Xontogeny	EIC Path Cell therapy sub-portfolio
Aera	$193M	2/16	Non-viral nucleic acid delivery platform	Arch, GV, Lux	EIC Path Gene therapy sub-portfolio

(1) BioCentury - The 20 biggest venture raises year-to-date: genetic medicines dominate

Takeaways from the European Health Summit and EHS Task Force Public debates on Gene Therapies

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Prof. Hildegard Buening, Professor and President of the European Society for Cell and Gene Therapies: "*It is our collective responsibility to make gene therapies available and accessible to those who are in need.*"

Claire Booth, Professor of gene therapies and paediatric immunology: "*When treating a rare disease, a collaboration from every stakeholder is vital, but we should also take into account the patients involvement.*"

Jo De Cock, Chair of the Belgian National Committee for Physicians and Health Insurance Funds: "*The fight for gene and cell therapies should be at the core of European and pharmaceutical strategies"*

Grant Holley, ASGCT's Director of Science and Education: "*Need to educate providers and the public about gene therapy and cell therapy treatments. The knowledge gap is everywhere because change is happening so fast*"

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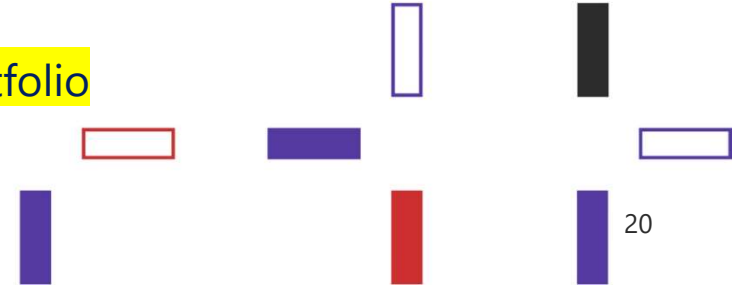

EIC is strategically positioned well with its 2021 CGT portfolio

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- President Biden launched a national biomanufacturing initiative with more than $2 bn (Dec, 2022)
- The EU Biotech and Biomanufacturing initiative: Priority area in health for 2024
- Bayern to open the first plant ($250M) to produce transformational cell therapies on a global scale
- Novo Nordisk Foundation (NNF) to establish a 160M cell manufacturing center (operational 2027)
- Harrington Discovery Institute, Ohio to invest €150M to Oxford University to advance treatments for RD with emphasis on CGT. The goal is to deliver 40 new therapies in the next 10 years
- The US FDA is now addressing the challenges of CGT manufacturing with the proposed draft guidance (Aug 2023): *(This guidance should reduce some of the uncertainties CGT sponsors face and help facilitate more meaningful discussions between FDA and industry on quality issues".*